Package leaflet: Information for the user

Mepsevii 2 mg/mL concentrate for solution for infusion
vestronidase alfa

▼This medicine is subject to additional monitoring. This will allow quick identification of new safety information. You can help by reporting any side effects you may get. See the end of section 4 for how to report side effects.

Read all of this leaflet carefully before you start using this medicine because it contains important information for you.
- Keep this leaflet. You may need to read it again.
- If you have any further questions, ask your doctor.
- This medicine has been prescribed for you only. Do not pass it on to others. It may harm them, even if their signs of illness are the same as yours.
- If you get any side effects, talk to your doctor. This includes any possible side effects not listed in this leaflet (see section 4).

What is in this leaflet

1. What Mepsevii is and what it is used for

What Mepsevii is
Mepsevii contains an enzyme called vestronidase alfa. This belongs to a group of medicines called enzyme replacement therapies. It is used in adults and children of all ages with MPS VII to treat non-neurological manifestations of the disease (mucopolysaccharidosis VII, also known as Sly Syndrome).

What is MPS VII
MPS VII is an illness that runs in families, where the body does not produce enough of an enzyme called beta–glucuronidase.
- This enzyme helps to break down sugars in the body called mucopolysaccharides.
- Mucopolysaccharides are made in the body and they help build bones, cartilage, skin, and tendons.
- These sugars are re-cycled all the time – new ones are made and old ones are broken down.
- Without enough beta–glucuronidase, parts of these sugars build up in cells, leading to damage in the body.

How Mepsevii works
This medicine replaces beta-glucuronidase – this helps to break down the sugars that collect in the tissues of people with MPS VII.
- Treatment may improve various signs and symptoms of illness, like walking difficulties and tiredness.

Starting treatment early in children may stop the illness getting worse and reduce permanent damage.

2. What you need to know before you are given Mepsevii

You must not be given Mepsevii
- If you have ever had a severe allergic reaction to vestronidase alfa or any of the other ingredients of this medicine (listed in section 6).

Warnings and precautions
Talk to your doctor before you are given Mepsevii.

The effects of treatment with vestronidase alfa should be periodically evaluated and discontinuation of treatment should be considered in cases where clear benefits (including stabilisation of disease manifestations) are not observed. Discontinuation of treatment may cause significant worsening of clinical status.

It should be considered that the administration of vestronidase alfa does not affect the irreversible complications (e.g. skeletal deformities).

Look out for side effects during or shortly after Mepsevii infusion

- You may have side effects while you are being given Mepsevii or for up to a day afterwards. These side effects are called infusion reactions because they are caused by the infusion (drip) of the medicine. They may include an allergic reaction (see section 4). If you have an infusion reaction, **tell your doctor straight away.**
- If you have an allergic reaction during your infusion your doctor may slow down, or stop your infusion. Your doctor may also give (or have given) you other medicines to manage the allergic reaction such as an antihistamine or corticosteroid or an antipyretic, a medicine to reduce fever.

Other symptoms to look out for

- If you have neck or back pain, feel numb in your arms or legs, or experience lack of control over passing water (urine) or stools, **tell your doctor straight away**. These problems can be signs of the illness and may be caused by pressure on your spinal cord.

Other medicines and Mepsevii
Tell your doctor if you are using, have recently used or might use any other medicines.

Pregnancy and breast-feeding
If you are pregnant or breast-feeding, think you may be pregnant or are planning to have a baby, ask your doctor for advice before using this medicine.

You will not be given Mepsevii if you are pregnant unless treatment is clearly necessary. Discuss with your doctor if the benefits of using Mepsevii are greater than the possible risks to your unborn baby. This is because there is no experience on the use of Mepsevii during pregnancy.

It is not known whether Mepsevii passes into breast milk, but transfer of the medication to your baby is not expected. Discuss with your doctor if the benefits of using Mepsevii are greater than the potential risk to your baby while breast-feeding.

Driving and using machines
Mepsevii is not likely to affect you being able to drive or use machines.

Mepsevii contains sodium
This medicine contains 17.8 mg sodium (main component of cooking/table salt) in each 5-mL vial, and is administered with sodium chloride 9 mg/mL as a diluent. Each vial dosed is therefore equivalent to 1.8% of the recommended maximum daily dietary intake of sodium for an adult. Take this into account if you are on a controlled sodium diet.

3. How Mepsevii is given

Treatment with Mepsevii should be started and monitored by your doctor.
- Your doctor or nurse will give Mepsevii to you by an infusion (drip) into a vein.
- The medicine has to be diluted before being given.
- Your doctor may give (or have given) you some medicines to manage the allergic reaction such as an antihistamine or corticosteroid or an antipyretic, a medicine to reduce fever.

Dose
The dose you will receive is based on how much you weigh.
- The recommended dose is 4 mg for each kg of body weight.
- The dose is given every two weeks through a drip into a vein (intra-venous infusion).
- Each infusion will be given over about 4 hours.

If you are given more Mepsevii than you should
Mepsevii is given to you and monitored by your doctor. He or she will check that the correct dose has been given and take action as needed.

If you have any further questions on the use of this medicine, ask your doctor.

4. Possible side effects

Like all medicines, this medicine can cause side effects, although not everybody gets them.

Side effects were mainly seen while patients were being given the medicine or within a day after the infusion (infusion reactions).

Serious side effects
Severe allergic reaction (Very common: may affect more than 1 in 10 people):
Tell your doctor or nurse immediately if you get any of the following symptoms of a severe allergic reaction (anaphylactoid reaction). The infusion will be stopped immediately and your doctor may give (or have given) you other medicines to manage the allergic reaction such as an antihistamine or corticosteroid or an antipyretic, a medicine to reduce fever. Symptoms of severe allergic reaction may include shortness of breath, wheezing, difficulty breathing, and swelling of the face and tongue.

Other side effects
Tell your doctor straight away if you notice any of the following side effects – you may need urgent medical treatment:

Very common side effects (may affect more than 1 in 10 people):
- Hives (urticaria)
- Rash
- Swelling at the infusion site including leaking into the tissue around the vein (infusion site swelling or infusion site extravasation)

Common side effects (may affect up to 1 in 10 people):
- Itching of the skin (pruritus)
- Loose stools (diarrhoea)
- Fever with involuntary contractions of muscles of face or limbs (febrile convulsion)
- Swelling around the infusion site

Reporting of side effects
If you get any side effects, talk to your doctor. This includes any possible side effects not listed in this leaflet. You can also report side effects directly via the:
Yellow Card Scheme

Website: www.mhra.gov.uk/yellowcard or search for MHRA Yellow Card in the Google Play or Apple App Store.
By reporting side effects you can help provide more information on the safety of this medicine.

5. How to store Mepsevii

Keep this medicine out of the sight and reach of children.

Do not use this medicine after the expiry date which is stated on the carton after 'EXP'. The expiry date refers to the last day of that month.

Unopened vials:
- Store in a refrigerator (2 °C to 8 °C).
- Do not freeze.
- Store in the original package in order to protect from light.
- Do not use this medicine if you notice particles.

Do not throw away any medicines via wastewater or household waste. Ask your pharmacist how to throw away medicines you no longer use. These measures will help protect the environment.

6. Contents of the pack and other information

What Mepsevii contains
- The active substance is vestronidase alfa. Each mL of concentrate contains 2 mg vestronidase alfa. Each vial of 5 mL concentrate contains 10 mg vestronidase alfa.
- The other ingredients are: sodium dihydrogen phosphate dihydrate, sodium chloride, histidine, polysorbate 20, and water for injections (for sodium, see section 2 under "Mepsevii contains sodium").

What Mepsevii looks like and contents of the pack
Mepsevii is supplied as a concentrate for solution for infusion (sterile concentrate). The colourless to slightly yellow concentrate must be free of visible particles. It is supplied in a clear glass vial with a rubber stopper and an aluminium seal with a plastic cap.

Pack size: 1 vial of 5 mL

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This medicine has been authorised under ‘exceptional circumstances’. This means that because of the rarity of this disease, it has been impossible to get complete information on this medicine.
The European Medicines Agency will review any new information on this medicine every year and this leaflet will be updated as necessary.

Other sources of information

Detailed information on this medicine is available on the European Medicines Agency web site: http://www.ema.europa.eu. There are also links to other websites about rare diseases and treatments.